

Supplementary Appendix: Stakeholder engagement in economic evaluation: Protocol for using the nominal group technique to elicit patient, healthcare provider, and health system stakeholder input in the development of an early economic evaluation model of chimeric antigen receptor T-cell therapy

Appendix I. *Stakeholder group discussion guides.*

Stakeholder Engagement in an Economic Evaluation of CAR T-cell Therapy
A research-driven nominal group discussion

Patient and patient family members

1. What are the *important costs/sacrifices* that come to mind for you when you think about a blood cancer treatment, such as CAR T-cell therapy?
2. What *benefits or potential treatment effects/outcomes* are important to you when considering different treatment options for blood cancer?

Clinicians

1. What *alternative treatment strategies (i.e., comparators)* for adults with relapsed/refractory ALL do you think should be added, removed, or changed in the CAR T-cell therapy economic evaluation model?
2. What *components/health states* of the CAR T-cell therapy economic evaluation model for adults with relapsed/refractory ALL do you think should added, removed, or changed based on your knowledge and expertise?
3. What *assumptions* does the CAR T-cell therapy economic evaluation model for adults with relapsed/refractory ALL make about treatment that you think should be added, removed, or changed based on your knowledge and expertise?

Payers and policy makers

1. What *alternative treatment strategies (i.e., comparators)* for adults with relapsed/refractory ALL do you think should be added, removed, or changed in the CAR T-cell therapy economic evaluation model?
2. What *assumptions* does the CAR T-cell therapy economic evaluation model for adults with relapsed/refractory ALL make about treatment that you think should be added, removed, or changed based on your knowledge and expertise?
3. What *project outputs* do you feel are necessary for effective dissemination and utilization of the proposed CAR T-cell therapy economic evaluation model?

Appendix II. Stakeholder discussion feedback form.**BEING INVOLVED IN EARLY ECONOMIC ANALYSES: YOUR FEEDBACK****Ranking Exercise**

Based on the discussion today, a number of suggestions were made to add to the economic model. Of these, we are interested in how important they are to you. Please rank the following list of potential inputs and outputs to be considered in the economic evaluation model of CAR T-cell therapy in order of importance from 1 - #, with 1 being the most important and # being the least important.

[include list of inputs/themes generated in stakeholder group discussions]

Your views on how you were and could be involved in providing your perspective on early economic evaluations

1. Which option listed below best reflects how you see yourself and your role in our discussions?
 - a. Patient
 - b. Caregiver/family member of a patient
 - c. Patient or caregiver advocacy organization
 - d. Clinician
 - e. Researcher
 - f. Healthcare payer
 - g. Healthcare policy maker
 - h. Other *[please describe]*
2. Using the definitions provided below, please select the response which best reflects your level of engagement in participating in the CAR T-cell therapy economic evaluation.
[Inform, Consult, Involve, Collaborate, Empower]

Definitions:

Inform: I was informed of the decisions after they had already been made by the research team.

Consult: I provided my thoughts, opinions, and views to the research team.

Involve: I worked with the research team to include my perspectives.

Collaborate: I shared control with the research team of decision-making processes.

Empower: I made final decisions, which were implemented by the research team.

3. How satisfied are you with your level of involvement in CAR T-cell therapy economic evaluation in the discussion? *[Very dissatisfied, Dissatisfied, Neutral, Satisfied, Very Satisfied]*

4. Please select the response which best reflects the level of influence (or effect) you feel your input had in contributing to the CAR T-cell therapy economic evaluation. [*Not at all influential, Slightly influential, Influential, Very influential, Extremely influential*]
5. In your opinion, was this level of engagement... [*Insufficient, Neutral, Sufficient, Above expectation*]
6. For each step of the CAR T-cell therapy economic evaluation process listed below, please select the response which best reflects the way in which you (or someone with your experience) might be interested in being involved in the future (select all that apply for each step of the process).

	Inform (being informed of decisions that were already made by the research team)	Consult (providing your views to the research team)	Involve (working with the research team to include your perspectives)	Collaborate (sharing control with the research team of decision-making processes)	Empower (making final decisions, which were implemented by the research team)
a. Planning and designing the study (e.g., setting project goals and objectives, determining research question and approach to data collection, etc.)					
b. Generating data: data, facts, logic, cross-checking (e.g., conducting research to collect and validate information)					
c. Identifying health economic model outputs (e.g., choosing the type of results produced from the evaluation, such as cost-minimization analysis, cost-benefit analysis, cost-effectiveness)					

<i>analysis, and cost-utility analysis, etc.)</i>					
d. Conducting the health economic evaluation (<i>e.g., including clinical evidence and the costs of treatment in the mathematical calculations of the health economic evaluation</i>)					
e. Analysing and validating the health economic evaluation (<i>e.g., consulting key stakeholders and experts to make sure the economic evaluation is accurate and reflects their lived experience/expertise</i>)					
f. Interpreting the health economic evaluation model results (<i>e.g., thinking about how the results of the economic evaluation may affect individuals, healthcare systems, and the delivery of care</i>)					
g. Sharing the health economic evaluation results (<i>e.g., sharing the results of the economic evaluation with different stakeholders to raise awareness and inform future research and development</i>)					

h. Implementing the health economic evaluation results (e.g., using the evaluation results to determine how treatments are funded)					
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7. The following is a list of factors that may or may not have affected your participation in the CAR T-cell therapy economic evaluation stakeholder group discussion, and that could affect your participation in similar processes in the future. Specifically, we are interested in which factors you feel may or may not have gotten or will get in the way of **you contributing your perspective on the CAR-T cell therapy economic evaluation**.

For each factor below, please indicate the degree to which you agree or disagree with the statement. Each factor is associated with a 5-point scale from strongly disagree to strongly agree. There are no right or wrong answers, we are interested in your honest opinion.

Factors	Strongly disagree	Disagree	Neutral	Agree	Strongly agree
I know enough about healthcare cost and benefit (i.e., economic) evaluations					
I am clear about the purpose of the CAR T-cell therapy economic evaluation					
I am prepared to contribute to the CAR T-cell therapy economic evaluation					
I am clear about my role in the CAR T-cell therapy economic evaluation					
I am confident in my ability to contribute to the CAR T-cell therapy economic evaluation					
I am clear about how the CAR T-cell therapy economic evaluation will benefit me					
My engagement in the CAR T-cell therapy economic evaluation will have an impact					
CAR T-cell therapy economic evaluation is a priority for me					

The stakeholder group dialogue created an environment that allowed me to participate					
The research team supported my engagement in the CAR T-cell therapy economic evaluation					
The research team valued my input in the CAR T-cell therapy economic evaluation					
Other individuals in the stakeholder group discussions supported my engagement in the CAR T-cell therapy economic evaluation					
Family and friends supported my engagement in the CAR T-cell therapy economic evaluation					
Putting a value on health/healthcare is emotional and challenging for me to discuss					
I do not have previous experience in health economic evaluation processes					

8. How much of an effect do you think participating in an online discussion instead of an in-person discussion had on your contribution to the stakeholder dialogue?
[No effect, minor effect, moderate effect, major effect]
9. If the online platform had an effect, what was the nature of this effect in terms of allowing you to contribute to the stakeholder group discussion?
[Very positive, positive, neutral, negative, very negative]
10. What were the advantages to you using an online platform for the stakeholder discussion?
[Open input]
11. What were the challenges to you using an online platform for the stakeholder discussion?
[Open input]
12. Please share any approaches or strategies that helped you to contribute to the stakeholder group discussion that might be helpful for others to be involved in the future.
[Open input]
13. Based on your experience, what recommendations do you have to improve stakeholder engagement in the future?
[Open input]

Appendix III. *Guidance for Reporting Involvement of Patients and the Public (GRIPP) 2 short form.*

Section and topic	Item	Reported on page No.
1: Aim	<p><i>Report the aim of patient and public involvement (PPI) in the study</i></p> <p>The research project includes a patient partner co-investigator on the multidisciplinary team grant to co-develop the research project and engage patients and the public in the conduct of an early economic evaluation. From their frequent interactions with fellow patients, investigators, clinicians, and the public, our patient partners identified a need to increase the involvement of the public, and more specifically patients and their caregivers, in health economic evaluation processes to ensure their perspectives are captured. The aim of PPI in this study is to inform and facilitate PPI in early economic evaluations.</p>	6-7
2: Methods	<p><i>Provide a clear description of the methods used for PPI in the study</i></p> <p>The research project was developed in close cooperation with our patient partner throughout several project meetings and with regular correspondence via email.</p> <p>Our patient partner contributed to and influenced all facets of the research project; this included collaboration in setting the research question, determining the study design, and informing the recruitment strategy, consent process, and planned analysis to produce meaningful results and limit the undue burden on potential participants.</p> <p>The research materials, including the recruitment materials, question guides, PowerPoint presentations, and Feedback form were developed iteratively with our patient partner to ensure accessibility and clarity for lay audiences. Additionally, the stakeholder discussions and research materials were piloted with patients and the public for additional feedback about the research approach, language, and user-friendliness.</p> <p>The stakeholder groups and the associated Feedback form planned and designed with our patient partner that are outlined in this study protocol are a means to inform future PPI strategies in early economic evaluations (this</p>	8-12

	engagement will be reported using the GRIPP2 in the subsequent results papers).	
3: Study results	<i>Outcomes – Report the results of PPI in the study, including both positive and negative outcomes</i> To be reported in subsequent results papers.	N/A
4: Discussion and conclusions	<i>Outcomes – Comment on the extent to which PPI influenced the study overall. Describe positive and negative effects</i> To be reported in subsequent results papers.	N/A
5: Reflections/critical perspective	<i>Comment critically on the study, reflecting on the things that went well and those that did not, so others can learn from this experience</i> To be reported in subsequent results papers.	N/A